Medical Drug Clinical Criteria

Subject:	Voxzogo (vosoritide)		
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Table of Contents

Overview

This document addresses the use of Voxzogo (vosoritide), a C type natriuretic peptide (CNP) analog approved by the Food and Drug Administration (FDA) to increase linear growth in pediatric individuals with achondroplasia who are 5 years of age and older with open epiphyses. The dose of Voxzogo is based on actual body weight and is administered via subcutaneous injection once daily. Growth and physical development should be monitored regularly every 3 to 6 months, and Voxzogo should be permanently discontinued upon closure of epiphyses.

The safety and efficacy of Voxzogo was evaluated in 121 pediatric individuals with achondroplasia in a 52-week, multi-center, randomized, double-blind, placebo-controlled phase 3 study. Study participants had genetically-confirmed achondroplasia and open epiphyses and were randomized to either Voxzogo or placebo. Voxzogo demonstrated a statistically significant improvement in annualized growth velocity (AGV) of 1.57 cm/year compared with placebo. Voxzogo was approved under an accelerated pathway based on an improvement in AGV, and continued approval may be contingent upon verification of clinical benefit (pubertal growth velocity, body segment proportionality, final adult height, achondroplasia-associated complications) in confirmatory trials.

Clinical Criteria

When a drug is being reviewed for coverage under a member's medical benefit plan or is otherwise subject to clinical review (including prior authorization), the following criteria will be used to determine whether the drug meets any applicable medical necessity requirements for the intended/prescribed purpose.

Voxzogo (vosoritide)

Initial requests for Voxzogo (vosoritide) may be approved if the following criteria are met:

I. Individual is 5 to 17 years of age; **AND**
II. Individual has a diagnosis of achondroplasia; **AND**
III. Documentation is provided that diagnosis has been verified via genetic mutation in fibroblast growth factor receptor 3 (*FGFR3*); **AND**
IV. Documentation is provided that individual has epiphyses that have not yet closed.

Continuation requests for Voxzogo (vosoritide) may be approved if the following criteria are met:

I. There is clinically significant improvement in growth velocity; **AND**
II. Documentation is provided that individual has epiphyses that have not yet closed.

Requests for Voxzogo (vosoritide) may not be approved for the following:

I. Individual with an eGFR <60 mL/min/1.73 m^2; **OR**
II. Individual using in combination with growth stimulants (growth hormone therapy, insulin-like growth factor 1 or anabolic steroids) (NCT 03197766); **OR**
III. Individual undergoing limb-lengthening surgery (NCT 03197766); **OR**
IV. May not be approved when the above criteria are not met and for all other indications.

Approval Duration
Initial Requests: 6 months
Continuation Requests: 12 months

Quantity Limits

Voxzogo (vosoritide) Quantity Limit

Drug	Limit
Voxzogo (vosoritide) 0.4 mg, 0.56 mg, 1.2 mg single-dose vial	1 vial per day

Coding

The following codes for treatments and procedures applicable to this document are included below for informational purposes. Inclusion or exclusion of a procedure, diagnosis or device code(s) does not constitute or imply member coverage or provider reimbursement policy. Please refer to the member's contract benefits in effect at the time of service to determine coverage or non-coverage of these services as it applies to an individual member.

HCPCS

J3490	Unclassified drugs (when specified as [Voxzogo] (vosoritide)
C9399	Unclassified drugs or biologicals (when specified as [Voxzogo] (vosoritide)

ICD-10 Diagnosis

All diagnoses pend

Document History

Revised: 2/24/2023
Document History:

- 2/24/2023 – Annual Review: Wording and formatting changes. Coding Reviewed: No changes.
- 2/25/2022 – Annual Review: Add new clinical criteria and quantity limit for Voxzogo. Coding Reviewed: Added HCPCS J3490, C9399. All diagnoses pend.

References

1. BioMarin Pharmaceutical. A Study to Evaluate the Efficacy and Safety of BMN 111 in Children with Achondroplasia. NLM Identifier: NCT 03197766. Last updated: March 2, 2022. Available at: https://www.clinicaltrials.gov/ct2/show/NCT03197766?term=03197766&draw=2&rank=1. Accessed: January 7, 2023.
2. DailyMed. Package inserts. U.S. National Library of Medicine, National Institutes of Health website. http://dailymed.nlm.nih.gov/dailymed/about.cfm. Accessed: January 7, 2023.
3. DrugPoints® System [electronic version]. Truven Health Analytics, Greenwood Village, CO. Updated periodically.
4. Lexi-Comp ONLINE™ with AHFS™, Hudson, Ohio: Lexi-Comp, Inc. Updated periodically.
5. Savarirayan R, Tofts L, Irving M, et. al. Once-daily, subcutaneous vosoritide therapy in children with achondroplasia: a randomised, double-blind, phase 3, placebo-controlled, multicentre trial. *Lancet*. 2020; 396(10252):684-692.

Federal and state laws or requirements, contract language, and Plan utilization management programs or polices may take precedence over the application of this clinical criteria.

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